Stem Cell Agency Board Invests in Therapy Targeting Deadly Blood Cancers

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Oakland, CA – Hematologic malignancies are cancers that affect the blood, bone marrow and lymph nodes and include different forms of leukemia and lymphoma. Current treatments can be effective, but in those patients that do not respond, there are few treatment options. Today, the governing Board of the California Institute for Regenerative Medicine (CIRM) approved investing $4.1 million in a therapy aimed at helping patients who have failed standard therapy.

Dr. Ezra Cohen, at the University of California San Diego, and Oncternal Therapeutics are targeting a protein called ROR1 that is found in B cell malignancies, such as leukemias and lymphomas, and solid tumors such as breast, lung and colon. They are using a molecule called a chimeric antigen receptor (CAR) that can enable a patient's own T cells, an important part of the immune system, to target and kill their cancer cells. These cells are derived from a related approach with an antibody therapy that targets ROR1-binding medication called Cirmtuzumab, also created with CIRM support. This CAR-T product is designed to recognize and kill cancer stem cells that express ROR1.

This is a late-stage preclinical project so the goal is to show they can produce enough high-quality cells to treat patients, as well as complete other regulatory measures needed for them to apply to the US Food and Drug Administration (FDA) for permission to test the therapy in a clinical trial in people.

If given the go-ahead by the FDA the therapy will target patients with chronic lymphocytic leukemia (CLL), mantle cell lymphoma (MCL) and acute lymphoblastic leukemia (ALL).

"CAR-T cell therapies represent a transformational advance in the treatment of hematologic malignancies," says Dr. Maria T. Millan, CIRM's President and CEO. "This approach addresses the need to develop new therapies for patients whose cancers are resistant to standard chemotherapies, who have few therapeutic options and a very poor chance or recovery."

The CIRM Board also approved investing $44,101,935 in 11 projects as part of our Translational Research Program (TRAN). The goal of this research is to support promising stem cell-based projects that
accelerate completion of translational stage activities necessary for advancement to clinical study or broad end use.

"Our goal is to always move the most promising research forward as fast as we can," says Dr. Millan. "That's why these programs are so important. They reflect potential therapeutic approaches that have shown promise in the lab and are ready to take the next step, to undergo further testing and examination to see if they might be able to work in patients."

The TRAN awards go to:

APP #	TITLE	APPLICANT	AMOUNT
TRAN1-12905	Development of novel chimeric antigen receptor (CAR) T cell therapy in patients with recurrent EGFRvIII+ glioblastom	H. Okada - UCSF	$4,556,536

TRAN4-13022	Human induced Pluripotent Stem Cell (iPSC)-derived micro-heart muscles for high-throughput cardiac drug discovery	S. Wall – Organos Inc.	$1,119,382
TRAN1-12911	Mucopolysaccharidosis type II: Plasma cell delivery of iduronate sulfatase	R. Hayes – Immusoft Corp.	$3,994,676
TRAN1-12895	Hematopoietic Stem Cell Gene Therapy for Immunodysregulation Polyendocrinopathy Enteropathy X-linked (IPEX) Syndrome	K. Masiuk - ImmunoVec	$3,551,332
TRAN1-12890	Clinical translation of human embryonic stem cell-derived protein therapy that positively regulates the regenerative capacity of post-natal muscle for treating myotonic dystrophy type 1 (DM1)	J. O'Connell - Juvena Therapeutics Inc	$3,906,376
TRAN1-12987	Chimeric Antigen Receptors (CAR) for the treatment of refractory pediatric B-cell acute lymphoblastic leukemia	A. Wiita - UCSF	$3,330,801
TRAN1-12893	Targeting stromal progenitors to prevent the development of heart failure	A. Deb - UCLA	$4,841,428
TRAN1-12920	Development of a gene editing therapy for Duchenne muscular dystrophy	C. Young - MyoGene Bio LLC	$3,400,000

TRAN1-13059	A human neural stem cell therapeutic candidate for the treatment of chronic cervical spinal cord injury	A. Anderson - UC Irvine	$5,552,611
TRAN1-12891	Clinical Translation of Allogenic Regenerative Cell Therapy for White Matter Stroke and Vascular Dementia	I. Llorente - UCLA	$5,925,602
TRAN1-12907	Investigational New Drug (IND)-enabling studies of a wearable therapeutic device for cardiac regeneration after myocardial infarction (MI)	P. RUIZ-LOZANO - Regencor, Inc.	$3,923,191

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov
